- **EudraCT ID:** 2021-000251-39
- **Takeda Study ID:** TAK-994-1504
- **Product:** TAK-994
- **Protocol Title:** A Dose-Blind Extension Study With Double-blind, Placebo-Controlled, Randomized Withdrawal Period to Evaluate the Safety and Explore the Pharmacokinetics and Pharmacodynamics of TAK-994 in Adults With Narcolepsy With Cataplexy (Narcolepsy Type 1)
- Trial Status: Prematurely Ended
- Rationale: A safety signal emerged in Phase 2 studies of TAK-994. As an immediate precautionary measure, Takeda has suspended dosing of patients and has decided to stop Phase 2 studies early.
- **Actual enrolled:** 26
- **Subject Disposition**

A total of 26 subjects were enrolled of the 43 subjects who completed TAK-994-1501 Part B. No subjects experienced screen failure.

A total of 26 subjects were randomized and received at least 1 dose of study drug: 8 subjects received TAK-994 low dose BID, 9 subjects received TAK-994 middle dose BID, and 8 subjects received TAK-994 high dose BID.

Overall, 5 subjects completed treatment with the study drug and completed the study visits. Of the subjects that discontinued, the most common reason for both discontinuation of the study drug and discontinuation from the study was study terminated by sponsor.

- **Baseline Characteristics**

In this study, overall mean age was 30.8 years; there were 12 men and 14 women. All subjects were non-Hispanic except 1 Hispanic or Latino subject. A total of 18 subjects were white, 3 were Black or African American, 3 were Asian, 1 was multiple race, and the race of 1 subject was not reported. Overall median height was 171.4 cm, weight was 77.5 kg, and BMI was 26.0 kg/m^2. Overall mean average sleep latency was 26.6 minutes, ESS score was 6.0, and WCR was 3.6.

- **Full Data Analysis**

Due to long term follow up of patients with serious hepatic safety events, database lock and full data analyses were significantly delayed. Analysis of the totality of data continues as does investigation of contributing factors to safety events and benefit/risk analysis of TAK-994. To allow the totality of the safety and efficacy data to be evaluated, and avoid any impact to its scientific integrity, full data will be posted by November 2023.